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## Hint of Success Indicated in Gene Therapy

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By NICHOLAS WADE

**G**ene therapy, a technique long on promise and so far very short on fulfillment, may be achieving a glimmering of success in a treatment for hemophilia B, a disease in which the blood does not clot properly.

The idea of gene therapy is to treat diseases that are the result of a defective gene by inserting the correct form of the gene into a patient's cells. If the technique worked, it could be a powerful remedy for many diseases that are hard to treat. But despite 20 years of effort and some 200 current trials, the Food and Drug Administration has yet to approve any form of gene therapy.

Biologists report in today's issue of the journal *Nature Genetics* that they gave patients suffering from hemophilia B the correct form of the gene for Factor IX, part of the cascade of proteins involved in forming a blood clot.

The trial involved only three patients and was designed to test the safety of the gene therapy, not its effectiveness. From earlier tests in mice and dogs, the researchers had calculated that the dose of the gene given the patients would be too low to offer any clinical benefit.

To their surprise, the researchers said they found that two of the patients were noticeably better, as judged by their lesser need to inject themselves for bleedings with extra Factor IX protein.

Both patients turned out to be making enough of their own Factor IX that clinically effective amounts were reaching the bloodstream, the researchers reported. The correct form of the gene had been injected into their muscle cells.

"When we first got the result, we said that must be a mistake," said Dr. Katherine A. High of Children's Hospital of Philadelphia, an author of the report.

Another author, Dr. Mark A. Kay of Stanford University, said, "The patient noticed it first, and we were real skeptical."

Although a safety trial can prove nothing about a treatment's effectiveness, these early results are encouraging because two of the three patients showed a benefit at the lowest of three planned doses.

"The data is preliminary, but it is quite suggestive that higher doses will confirm these results are real," said Dr. Arthur Beaudet, a geneticist at the Baylor College of Medicine. "I think these investigators are really close."

Dr. Beaudet, who was not involved in the new study, said he thought that proof that the therapy worked was a year or so away. How soon a treatment would be widely available, he said, depends on factors like action by patient advocate groups and the availability of the virus used to insert the corrective gene.

About 3,300 people in the United States have hemophilia B, caused by one of several possible mutations in the gene for Factor IX. Another 12,000 people suffer from hemophilia A, the result of mutations in the gene for Factor VIII, also a vital component of the body's blood-clotting system.

Dr. High and Dr. Kay have each been trying separately to develop gene therapy treatments for hemophilia for more than a decade but have now joined forces.

The promising result of their research comes in the wake of gene therapy's long record of failures and the recent death of a patient, Jesse Gelsinger, 18, in a gene therapy trial at the University of Pennsylvania. Mr. Gelsinger died after receiving an injection into the liver of a high dose of adenovirus, one of the causes of the common cold.

The team led by Dr. Kay and Dr. High used a different virus, adeno associated virus, which has very few genes of its own and depends on adenovirus to reproduce itself.

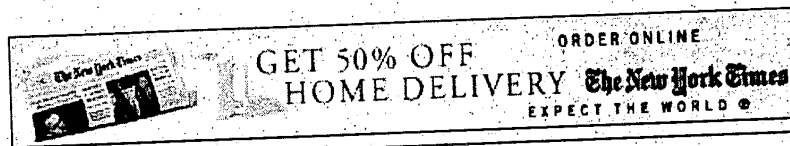
Viruses are used to deliver the corrective human genes in most gene therapy trials because of their natural ability to enter human cells and incorporate their genetic material into human chromosomes. The adeno associated virus used by Dr. High and Dr. Kay is not as provocative to the immune system as adenovirus and the cells it

infects survive much longer. Dr. High said that dogs given the Factor IX gene nearly three years ago were still producing the gene's protein.

The treatment may be working better in people than it did in animals, she said, because the adeno associated virus is a human virus and may be more active in its natural host. One drawback of the virus is that it can carry only rather small genes. The human gene for Factor VIII, for example, is too large for it, so some other virus must be found to treat hemophilia A.

Dr. High said gene therapy's record of failure was due largely to the difficulty of finding appropriate viruses for gene delivery.

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